



New positive donidalorsen data to be presented at 2024 American College of Allergy, Asthma & Immunology (ACAAI) Annual Scientific Meeting

October 3, 2024

- *Data continue to demonstrate donidalorsen potential to deliver significant and sustained reductions in HAE attacks up to three years with monthly or every two-month dosing*

CARLSBAD, Calif., Oct. 3, 2024 /PRNewswire/ -- [Ionis Pharmaceuticals, Inc.](#) (Nasdaq: IONS) announced today that it will present new three-year data from the Phase 2 open-label extension (OLE) study as well as additional results from the pivotal Phase 3 OASIS-HAE and OASISplus studies of donidalorsen, the company's investigational RNA-targeted prophylactic medicine for hereditary angioedema (HAE). Results will be presented at the 2024 American College of Allergy, Asthma & Immunology (ACAAI) Annual Scientific Meeting in Boston, Massachusetts.



New data at ACAAI demonstrate donidalorsen significantly reduced HAE attacks, translating to high levels of disease control and significant and clinically meaningful improvements in quality-of-life across multiple measures in a vast majority of patients, sustained up to three years.

All presentations can be found on Ionis' [website](#), including:

- **Phase 2 Open-Label Extension of Donidalorsen In Patients with Hereditary Angioedema: A Week 197 Analysis**
 - e-Poster Presentation: October 25, 2024 (Monitor 21, Exhibit Hall A)
 - Presenting Author: Laura Bordone
- **Impact of Donidalorsen on Patient-Reported Outcomes: Results from the Phase 3 OASIS-HAE Study**
 - e-Poster Presentation: October 25, 2024 (Monitor 21, Exhibit Hall A)
 - Presenting Author: Aaron Yarlus
- **Treatment Of Hereditary Angioedema: Safety, Efficacy, and Patient Preference after Switching to Donidalorsen (OASISplus Study)**
 - e-Poster Presentation: October 25, 2024 (Monitor 21, Exhibit Hall A)
 - Presenting Author: Danny Cohn
- **Donidalorsen for Hereditary Angioedema: Results from the OASISplus Open-Label Extension Study**
 - e-Poster Presentation: October 25, 2024 (Monitor 21, Exhibit Hall A)
 - Presenting Author: Raffi Tachdjian
- **Donidalorsen Exposure-Response Analysis: Hereditary Angioedema Attack Rate versus Plasma Prekallikrein Concentration Relationship**
 - e-Poster Presentation: October 25, 2024 (Monitor 19, Exhibit Hall A)
 - Presenting Author: Pratap Singh
- **Drug Patterns, Clinical Outcomes, and Economic Costs among Hereditary Angioedema Patients Initiating Long-Term Prophylaxis**
 - e-Poster Presentation: October 25, 2024 (Monitor 19, Exhibit Hall A)
 - Presenting Author: William R. Lumry

Ionis previously [reported](#) positive results from the Phase 3 OASIS-HAE and OASISplus studies. Data from both studies were presented at the 2024 European Academy of Allergy and Clinical Immunology (EAACI) Annual Congress in Valencia, Spain and results from OASIS-HAE were published in *The New England Journal of Medicine*. The company also previously [reported](#) positive two-year results from the Phase 2 OLE study last year.

About Hereditary Angioedema (HAE)

HAE is a rare and potentially life-threatening genetic condition that involves recurrent attacks of severe swelling (angioedema) in various parts of the body, including the hands, feet, genitals, stomach, face and/or throat. HAE is estimated to affect more than 20,000 patients in the U.S. and Europe. In the U.S., doctors frequently use prophylactic treatment approaches to prevent and reduce the severity of HAE attacks in patients.

About Donidalorsen

Donidalorsen is an investigational RNA-targeted medicine designed to target prekallikrein (PKK), which plays an important role in activating inflammatory mediators associated with acute attacks of hereditary angioedema (HAE). By reducing the production of PKK, donidalorsen could be an effective prophylactic approach to preventing HAE attacks, if approved.

Donidalorsen is an investigational medicine that has not been approved for the treatment of any disease by regulatory authorities.

About Ionis Pharmaceuticals, Inc.

For three decades, Ionis has invented medicines that bring better futures to people with serious diseases. Ionis currently has five marketed medicines and a leading pipeline in neurology, cardiology, and other areas of high patient need. As the pioneer in RNA-targeted medicines, Ionis continues to drive innovation in RNA therapies in addition to advancing new approaches in gene editing. A deep understanding of disease biology and industry-leading technology propels our work, coupled with a passion and urgency to deliver life-changing advances for patients. To learn more about Ionis, visit [ionis.com](https://www.ionis.com) and follow us on [X \(Twitter\)](#) and [LinkedIn](#).

Forward-looking Statements

This press release includes forward-looking statements regarding Ionis' business and the therapeutic and commercial potential of our commercial medicines, donidalorsen, additional medicines in development and technologies. Any statement describing Ionis' goals, expectations, financial or other projections, intentions or beliefs is a forward-looking statement and should be considered an at-risk statement. Such statements are subject to certain risks and uncertainties including those inherent in the process of discovering, developing and commercializing medicines that are safe and effective for use as human therapeutics, and in the endeavor of building a business around such medicines. Ionis' forward-looking statements also involve assumptions that, if they never materialize or prove correct, could cause its results to differ materially from those expressed or implied by such forward-looking statements. Although Ionis' forward-looking statements reflect the good faith judgment of its management, these statements are based only on facts and factors currently known by Ionis. Except as required by law, we undertake no obligation to update any forward-looking statements for any reason. As a result, you are cautioned not to rely on these forward-looking statements. These and other risks concerning Ionis' programs are described in additional detail in Ionis' annual report on Form 10-K for the year ended December 31, 2023, and most recent Form 10-Q, which are on file with the Securities and Exchange Commission. Copies of these and other documents are available from the Company.

In this press release, unless the context requires otherwise, "Ionis," "Company," "we," "our" and "us" all refer to Ionis Pharmaceuticals and its subsidiaries.


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